Sickle Cell Disease Research, Surveillance, Prevention, and Treatment Act of 2017 (HR 2410, 115th Congress)

The Policy

What it does

Amends the Public Health Service Act to authorize sickle cell surveillance, prevention, and treatment initiatives and to establish conditions for collaboration with community-based organizations on such initiatives.

Synopsis

The Sickle Cell Disease Research, Surveillance, Prevention, and Treatment Act of 2017 (HR 2410) is designed to reauthorize expired federal sickle cell disease programs and establish new federal sickle cell disease research, surveillance, prevention, and treatment programs.

HR 2410 amends section 399V of title III of the Public Health Service Act (42 U.S.C. 280g-11 et seq.) to authorize the Department of Health and Human Services to undertake the following research and surveillance efforts:

- Establish research efforts to explore causes and cures for the disease.
- Award grants to entities in up to 20 states that demonstrate collaboration with other communal organizations and healthcare providers to:
  - Collect data on the prevalence and demographics of sickle cell disease;
  - Organize public health initiatives that increase access to health care, increase knowledge of recommended prevention and screening strategies, increase knowledge of recommended treatment, promote consistent guidelines for medical care, train healthcare providers and organizations, train state health departments, and educate communities at large; and
  - Research potential prevention and treatment strategies, particularly focusing on factors that contribute to the cause of the disease (including genetic factors), determine disease disparities, and affect disease outcomes.

HR 2410 also amends the American Jobs Creation Act of 2004 to reauthorize funding for the treatment program outlined in Section 712(c) (42 U.S.C. 300b-1 note), also known as the Sickle Cell Disease Treatment Demonstration Program, which attempts to coordinate sickle cell disease treatment efforts. The amendment reauthorizes the program and makes the following alterations to the program’s jurisdiction:

- Award grants to up to 25 entities per year;
- Expand resources for adolescents with sickle cell disease; and
The Science

Consequences:

Blood cells with normal hemoglobin are round, giving them a flexibility that allows them to circulate through blood vessels of all sizes and transport oxygen throughout the body. The hemoglobin protein is comprised of four protein subunits, two of which are called beta-globin and are produced by the HBB gene.[37] Each person has two HBB genes, one inherited from each parent. Some mutations in the HBB gene produce an abnormal beta-globin that functions improperly. For people who develop SCD, both HBB genes are thus mutated and at least one produces an abnormal beta-globin called hemoglobin S (HbS). The second HBB mutation also produces an abnormal beta-globin, either HbS or another variant like hemoglobin C (HbC), D (HbD), E (HbE), O (HbO), or ε (Hbε). This variable second mutation[41] defines the distinction between various forms of SCD (HbS).[42] The most severe form of SCD, sickle cell anemia (HbSS), occurs when individuals inherit copies of the HbS gene from both parents, and thus are only able to produce hemoglobin S.

According to the National Heart, Lung, and Blood Institute of the NIH[43], inheriting mutated hemoglobin genes alters the functional capacity of red blood cells. Red blood cells with normal hemoglobin are round, giving them a flexibility that allows them to circulate through blood vessels of all sizes and transport oxygen throughout the body. Abnormal hemoglobin can stick together to form long rods that cause red blood cell to be sickle shaped and inflexible. This inflexibility has two primary consequences:

- Less blood flow: Blood cells are more easily caught on the sides of blood vessels, either slowing the flow of blood or stopping it entirely. The lack of red blood cells in parts of the body can cause:
  - Acute and chronic pain;
  - Swelling;
  - Delayed growth; and
  - Organ complications, particularly in the liver, kidney, heart, and eyes.

Policy History

HR 2410 is the third version of the Sickle Cell Disease Research, Surveillance, Prevention, and Treatment Act. The bill was previously introduced as the Sickle Cell Disease Research, Surveillance, Prevention, and Treatment Act of 2014[44] (HR 5124[45], 113th Congress). A nearly identical bill was then introduced as the Sickle Cell Disease Research, Surveillance, Prevention, and Treatment Act of 2015[46] (HR 1807[47], 114th Congress). Representative Danny Davis (D-IL-7) introduced all three versions of the bill in the House.

The Science

Science Synopsis

Sickle cell disease (SCD)[32] describes a group of genetic blood disorders.[33] According to the Centers for Disease Control and Prevention (CDC)[34] around 100,000 Americans have SCD. The CDC further explains that those with SCD are primarily of African descent but that SCD also affects those from South America, Central America, the Caribbean, and the Mediterranean.

The National Institute of Health (NIH) explains[35] that SCD affects red blood cells by affecting hemoglobin[36], the protein in red blood cells[37] that transports oxygen through the body. The hemoglobin protein is comprised of four protein subunits[38], two of which are called beta-globin and are produced by the HBB gene[39]. Each person has two HBB genes, one inherited[40] from each parent. Some mutations in the HBB gene produce an abnormal beta-globin that functions improperly. For people who develop SCD, both HBB genes are thus mutated and at least one produces an abnormal beta-globin called hemoglobin S (HbS). The second HBB mutation also produces an abnormal beta-globin, either HbS or another variant like hemoglobin C (HbC), D (HbD), E (HbE), O (HbO), or ε (Hbε). This variable second mutation[41] defines the distinction between various forms of SCD[42]. The most severe form of SCD, sickle cell anemia (HbSS), occurs when individuals inherit copies of the HbS gene from both parents, and thus are only able to produce hemoglobin S.

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- Less blood flow: Blood cells are more easily caught on the sides of blood vessels, either slowing the flow of blood or stopping it entirely. The lack of red blood cells in parts of the body can cause:
  - Acute and chronic pain;
  - Swelling;
  - Delayed growth; and
  - Organ complications, particularly in the liver, kidney, heart, and eyes.
• **Anemia** [44]: Blood cells have a shorter life span because they burst easily. The lifespan of sickle cells is 10 to 20 days instead of the normal 90 to 120 days, making it difficult for the body to produce enough blood cells to keep up with blood cell turnover rate.

• **Other complications** [43] include increased infections, liver failure, brain development problems, kidney problems, heart disease. *priapism* [45], *gallstones* [46], *ulcers* [47], delayed puberty, and pregnancy problems.

Mutations in HBB are the primary cause of SCD. However, as evidenced by the diversity in symptoms between people with the same mutations, other genetic factors are likely to play a less direct role as modifiers of the disease. Many of these genetic factors are unknown or modify SCD through unknown pathways. Identifying and understanding the heritable genetic factors that affect expression of SCD could lead to improvements in treatments and general health care.

**Relevant Experts**

Soheir Saeed Adam, MBBCh [48], is an Assistant Professor of Medicine at the Duke University School of Medicine. Her research focuses on physical markers of sickle cell disease.

**Relevant Publications:**


Regina Denise Crawford, MD [54], is an Assistant Professor of Medicine at the Duke University School of Medicine. Her research involves studying the cognitive functions of those with sickle cell disease.

**Relevant Publications:**


Marilyn Jo Telen, MD [58], is a Professor of Medicine and Associate Professor of Pathology at the Duke University School of Medicine. She is also Director of the Duke Comprehensive Sickle Cell Center. Her research involves studying the adhesion receptors of cell membrane proteins to understand the interaction of these proteins with red blood cells and other proteins in sickle cell disease.

"Despite a vastly improved understanding of how sickle hemoglobin causes disease, slow progress has been made in development of new effective therapies, including medicines, bone marrow transplantation and gene therapy, the latter two being the only ones offering a potential cure for the disease. However, in the last few years, significant progress has been made on all three fronts, so that now is the time to truly push forward to perfect and complete these therapies. Two drugs have shown promise in early clinical trials, bone marrow transplantation has apparently cured small number of patients, and at least one patient has responded to gene therapy. However, given the low numbers affected by SCD in the US, pharmaceutical and biotech companies have a limited financial incentive to develop such therapies."

**Relevant Publications:**


**The Debate**

**Endorsements & Opposition**

Endorsements:

- **Representative Michael C. Burgess, MD** [62] explains how HR 2410 achieves the foundational necessity of increasing public awareness in a public statement [63]: "Having cared for patients with sickle cell disease as a physician at Parkland Hospital, I've seen firsthand the devastating effect this disease can have on people. This bill provides an important step forward in ensuring that we have the resources to better understand this disease and to maintain access to services for those affected by sickle cell disease."

- **Sonja Banks** [64], President of the Sickle Cell Disease Association of America, a national organization working to promote research and education on sickle disease, expressed support for a previous iteration of the bill (HR 1807[31], 114th Congress) in a testimony [65] in front of the Energy and Commerce Subcommittee on Health [66]. Her testimony brought three main points of support to attention:

  - "The treatment and prevention component reauthorization, contained within section 4 of the bill, sets a more realistic number of eligible entities which can be funded. The original law specified 40 eligible entities, H.R. 1807 set that number to 25 eligible entities."

  - "Importantly, a major advancement made in H.R. 1807 would place a duty on these grantees to "expand, coordinate, and implement transition services for adolescents with sickle cell disease making the transition to adult-focused health care...This very important change would make it a requirement for grantees to adopt strategies to ensure that these individuals transition appropriately, minimizing the disruption of care and resulting in better health outcomes."

  - "The current surveillance conducted by the CDC is limited to the state of California and the data collected is general in nature. The data which would be accumulated under this grant program authorized by HR 1807 would cover associated health outcomes, complications and treatments, and would result in public health initiatives and strategies which would improve current estimates about the incidence and prevalence of the disease, would identify health disparities, would assess the utilization therapies and strategies to prevent complications from the disease, and would evaluate the impact of genetic, environmental, behavioral and other risk factors that may impact health outcomes"
Opposition:

At present, there has not been any publicly reported opposition to this bill.

Status

HR 2410 was introduced in the House on May 11, 2017 and referred to the Committee on Energy and Commerce. After referral to and consideration by the Committee, it was ordered to be sent to the House floor without amendments on June 7, 2017. On February 26, 2018, the House debated and passed the still unamended bill by a voice vote; the bill was introduced into the Senate on February 27, 2018.

On February 28, 2018, Senator Tim Scott (R-SC) introduced the Sickle Cell Disease Research, Surveillance, Prevention, and Treatment Act of 2018, which is nearly identical to the version of 2410 passed by the House on February 26, 2018.

Recommended Citation


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[29] https://www.congress.gov/bill/114th-congress/senate-resolution/592?text&q;=7B%22search%22%3A%5B%22sickle+cell%22%5D%7D%26amp%3Bqr=1&q;=Sickle+Cell+Disease
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